

## Accelerating Medicines Partnership: Autoimmune Diseases of Rheumatoid Arthritis and Lupus

The National Institutes of Health (NIH), 10 biopharmaceutical companies, and several non-profit organizations have designed an unprecedented new partnership. Managed through the Foundation for the NIH (FNIH), the Accelerating Medicines Partnership (AMP) brings high-level government, industry, and non-profit foundation partners together to identify and validate the most promising biological targets of disease for new diagnostic and drug development. The partners have designed a bold milestone-driven research plan to tackle the challenge for the autoimmune diseases of **rheumatoid arthritis and systemic lupus erythematosus (lupus)**, as well as for Alzheimer's disease and type 2 diabetes. One of the features that makes this public-private partnership unique is that AMP data will be publicly accessible to the broad biomedical community for further research. This fact sheet addresses the AMP research plan for rheumatoid arthritis and lupus.

### Rheumatoid Arthritis and Lupus

Rheumatoid arthritis (RA) and lupus are relatively common, severe disorders. About 1.5 million people or about 0.6 percent of the U.S. adult population have rheumatoid arthritis. Estimating how many people in the U.S. have lupus is difficult because symptoms vary widely and onset is often hard to pinpoint. Both conditions are autoimmune diseases that occur when the immune system mistakenly attacks parts of the body that it is designed to protect. They represent just two of a larger number of autoimmune disorders, including multiple sclerosis, Crohn's disease, ulcerative colitis, type 1 diabetes, and psoriasis. These diseases share common flaws in immune function and regulation, leading to inflammation that destroys tissues. They can last a lifetime, cause severe disability, greatly affect quality of life, and are associated with increased risk of death.

### Need for New Therapies

Treatments for these diseases typically have been aimed at decreasing inflammation and pain by slowing down tissue damage and reducing complications. Most people with RA have only a partial response to available drugs, and many only respond to drugs for a limited period of time. In the case of lupus, no effective targeted therapies exist for the most severe forms of the disease. Research is needed to better understand the underlying disease process and identify parts of the immune system that aren't functioning correctly. This insight will help reveal the most promising new biological targets for drug development and match existing drugs to patients with specific molecular profiles who are most likely to benefit.

#### AMP Rheumatoid Arthritis and Lupus: *The Partners*

##### Government

- NIH

##### Industry

- AbbVie
- Bristol-Myers Squibb
- Merck
- Pfizer
- Sanofi
- Takeda

##### Non-Profit Organizations

- Foundation for the NIH
- Lupus Foundation of America
- Rheumatology Research Foundation

## AMP Approach

This project will analyze tissue and blood samples from people with RA and lupus to pinpoint genes, proteins, chemical pathways, and networks involved at a single cell level. This type of modular, molecular analysis will allow comparisons across the diseases and will provide insights into key aspects of the disease process. The project will identify differences between those RA patients who respond to therapies and those who do not, as well as provide a better systems level understanding of disease mechanisms in both RA and lupus. This knowledge is essential for the development of targeted therapies and for the application of existing and future therapies to appropriate patient populations.

The partnership will integrate several new or developing technologies to analyze single cells and groups of cells involved in autoimmunity in new ways; collect tissue samples, including synovium (the tissue that lines joints) from people with RA and lupus for molecular analysis; develop computational tools to integrate different data types to characterize molecular pathways; and make the data available to the broad research community for further analysis.

## Governance

The steering committee (SC) for RA and lupus will be comprised of representatives from NIH, FNIH, and participating companies and patient advocacy organizations. After AMP research grant awards are made, investigators carrying out the research will be added to the disease SCs. The SC is responsible for defining the research agenda and project plans, reviewing ongoing projects, and assessing the milestones. The SC will operate under the direction of the overall AMP Executive Committee comprised of representatives from NIH, FNIH, participating companies, the Food and Drug Administration, and patient advocacy organizations.

## Timeline and Deliverables

The research plan proposes a five-year program starting in late 2014. Year one will include startup activities such as validation of tissue acquisition processes and analytic technologies, and development of operating procedures. The second year will focus on identification of disease-specific pathways by comparing data from patients and healthy individuals. Years three-five will expand the scale to include comparisons of different subsets of patients with RA or lupus to allow molecularly based patient stratification for precise treatment. The final 12 months (2019) will also include preliminary target validation. The data will be made publicly available through an internet-based information portal.

## Budget: 5 years (\$41.6 Million Total Project Funding)

(\$Millions)	Total Project	Total NIH	Total Industry
<b>Rheumatoid Arthritis and Lupus</b>	41.6	20.9	20.7